

SMA Adult Outcomes Delphi Process Round 2 Report

The second round of the Delphi Process for SMA in Adults has been completed. Below includes figures and tables which summarize the findings of this round. In keeping with the data from the first round, we have included assessments for the averages and confidence intervals for each of the included outcomes.

Once again, there were 10 responses to the second round. The results have been compiled below. Figure 1 shows that the majority of responses were received from neurologists, with the remainder of responses being from physiatry. Figure 2 shows that responses were obtained from 6 provinces. Table 1 outlines the perceived clinical utility of each of the outcomes included in the survey and categorizes the results into each of the three primary groups of patients (non-sitters, sitters or ambulators). Table 2 describes the results in a categorical method, which indicates which outcomes met various cut-offs within each group of patients. The cut offs used are the lower bounds of the confidence interval being above values of 3 and 3.5, as well as an overall average above 3, 3.5, or 4. Included in table 2 is also a rating of current clinical use which indicates the number of respondents which currently utilize these outcomes in practice and how many respondents rated the outcome as being used often to always.

Next Steps

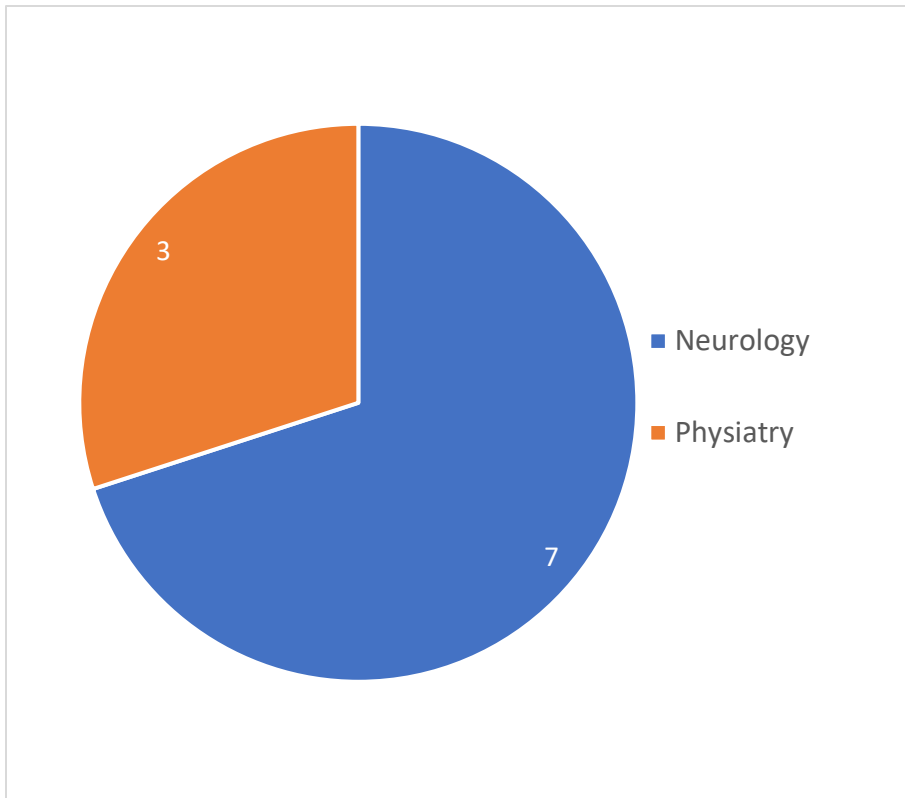
To follow up with this report, a meeting invite will be sent to discuss these results and has a goal of determining a final consensus on outcomes. With the conclusion of the consensus meeting, the goal will be to have reached a consensus on outcomes to be used in Adult SMA.

Relevant information which is available and will be sent to those attending the meeting is the table of outcome measures, Round 1 report, as well as the Round 2 report. Each of these documents have been previously distributed but will be distributed again ahead of time.

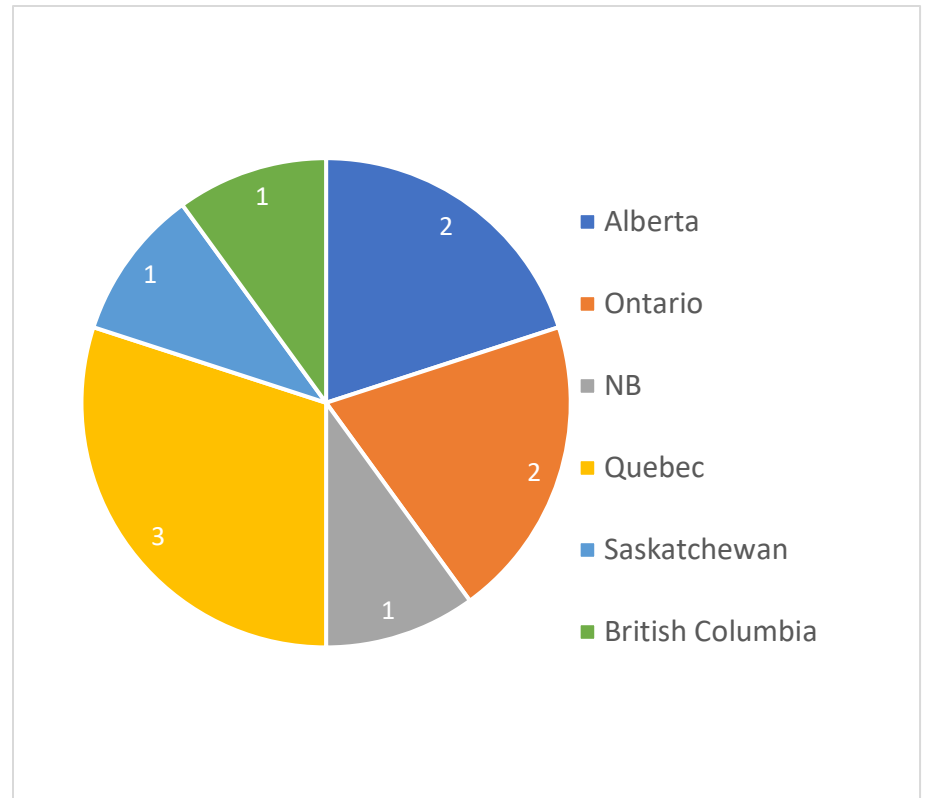
Table 1 – Round 2 Outcome Ratings of Clinical Utility and broken into three patient populations. Rated from 1 (Very Weak) to 5 (Very Strong)

	Non-Sitters			Sitters			Ambulators		
	Count (N)	Average [STD]	Confidence Interval	Count (N)	Average [STD]	Confidence Interval	Count (N)	Average [STD]	Confidence Interval
Gross Motor Function Measure (GMFM)	8	2.75 [1.28]	2.71-2.79	8	3.5 [1.31]	3.46-3.54	8	3.38 [1.30]	3.33-3.42
Hammersmith Function Motor Scale Expanded (HFMSSE)	10	3.3 [0.95]	3.28-3.319	10	4.3 [0.48]	4.29-4.31	10	4 [0.82]	3.98-4.02
Revised Hammersmith Scale (RHS)	10	3.6 [0.84]	3.58-3.62	10	4.4 [0.52]	4.39-4.41	10	4 [0.67]	3.99-4.01
Motor Function Scale (MFS)	7	3.43 [0.78]	3.40-3.46	7	3.57 [0.98]	3.53-3.61	7	3.71 [0.76]	3.68-3.75
Egen Klassifikation Scale Revisited (EK2)	6	3.83 [0.41]	3.81-3.86	6	3.5 [0.84]	3.45-3.55	6	2.33 [1.03]	2.28-2.39
Spinal Muscular Atrophy Functional Composite Scale (SMAFCS)	7	2.43 [1.13]	2.38-2.47	6	3 [1.41]	2.92-3.08	6	3.17 [1.47]	3.08-3.25
CHOP-ATEND	8	4 [1.41]	3.96-4.04	9	3.56 [1.24]	3.53-3.59	9	2.56 [1.42]	2.52-2.59
Revised Upper Limb Module (RULM)	10	3.90 [1.20]	3.88-3.92	10	4.1 [0.74]	4.09-4.11	10	3.4 [1.26]	3.37-3.43
10 Meter Walk Test (10MWT)	6	1 [0]	*	9	1.33 [1]	1.31-1.36	9	4.11 [0.93]	4.09-4.13
Timed Up and Go (TUG)	7	1 [0]	*	9	1.33 [1]	1.31-1.36	10	4.2 [0.92]	4.18-4.22
6 Minute Walk Test (6MWT)	8	1.38 [1.06]	1.34-1.41	9	1.33 [1]	1.31-1.36	10	4.4 [0.70]	4.39-4.41
2 Minute Walk Test (2MWT)	8	1.25 [0.71]	1.23-1.27	9	1.33 [1]	1.31-1.36	9	4 [0.5]	3.99-4.01
SMA Functional Rating Scale (SMAFRS)	9	3.89 [0.93]	3.87-3.91	10	4. [1.05]	3.98-4.02	10	3.9 [0.88]	3.88-3.92
Handheld Dynamometry (MVICT)	10	3 [0.82]	2.98-3.02	10	3.1 [0.99]	3.08-3.12	10	3.1 [0.99]	3.08-3.12
Forced Vital Capacity (FVC)	10	4.3 [0.67]	4.29-4.31	10	4.4 [0.70]	4.39-4.41	10	4 [0.67]	3.99-4.01
Peak Cough Flow (PCF)	10	4.3 [0.67]	4.29-4.31	10	4.4 [0.70]	4.39-4.41	10	3.9 [0.88]	3.88-3.92
Maximum Expiratory Pressure (MEP)	10	4.1 [0.57]	4.09-4.11	10	4.2 [0.63]	4.19-4.21	10	3.7 [0.67]	3.69-3.71
Maximum Inspiratory Pressure (MIP)	10	4 [0.47]	3.99-4.01	10	4.1 [0.57]	4.09-4.11	10	3.7 [0.67]	3.69-3.71
PedsQL/ PedsQL Neuromuscular Module	8	2.75 [0.89]	2.72-2.78	8	3 [1.20]	2.96-3.04	7	2.71 [1.38]	2.66-2.77
Short Form 36 (SF-36)	8	2.75 [0.71]	2.73-2.77	8	2.88 [0.99]	2.84-2.91	7	3.14 [1.21]	3.09-3.19
Sickness Impact Profile 68 (SIP-68)	7	2.43 [0.53]	2.41-2.45	7	2.57 [0.98]	2.53-2.61	7	2.86 [1.21]	2.81-2.91

*Indicates no Confidence Interval



**Figure 1 – Demographic Breakdown of Respondents;
by Specialty of Practice**



**Figure 2 – Demographic Breakdown of Respondents; by
Location**